Prevail Therapeutics Provides Program Update on PR001 in Parkinson's Disease with GBA1 Mutations and Neuronopathic Gaucher Disease

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NEW YORK, Sept. 10, 2019 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (NASDAQ: PRVL), a biotechnology company developing AAV-based gene therapies for patients with neurodegenerative disorders, today provided an update on the Company's clinical development of PR001, a potentially disease-modifying, single-dose gene therapy in development for Parkinson's disease with *GBA1* mutations (PD-GBA) and neuronopathic Gaucher disease (nGD).

PR001 for Treatment of PD-GBA

Prevail has an open Investigational New Drug Application (IND) for PR001 for the treatment of PD-GBA, the first indication the Company is pursuing. The Company is in the process of activating clinical sites for its Phase 1/2 clinical trial in PD-GBA and is on track to initiate patient dosing this year. Approximately 7%-10% of Parkinson's disease patients harbor an underlying mutation in the *GBA1* gene, and it is estimated that there are 90,000 PD-GBA patients in the U.S. alone. No therapies currently available have shown the ability to slow or stop the disease progression of PD-GBA. This randomized, double-blind, sham procedure-controlled, ascending dose Phase 1/2 clinical trial will enroll up to sixteen PD-GBA patients. Two escalating dose cohorts are planned. In each cohort, six patients will receive PR001, administered as a single injection, and two patients will receive a sham procedure as control. The trial will investigate the safety and tolerability of PR001 and will also measure key biomarkers and exploratory efficacy endpoints.

PR001 for Treatment of Pediatric nGD

Prevail is also developing PR001 for pediatric nGD, a second indication with urgent unmet need. The Company submitted an IND to the U.S. Food and Drug Administration (FDA) for PR001 for the treatment of pediatric patients with nGD. Following discussions with the FDA, and based on preclinical studies that demonstrated increased efficacy at a higher dose, Prevail is modifying the design of the Phase 1/2 clinical trial in nGD to commence at a dose higher than originally proposed. Prevail's IND for PR001 for the treatment of pediatric nGD has been placed on clinical hold pending FDA review of an amendment to the nGD IND, which will detail this modification. No safety or adverse events have been observed in any of Prevail's studies of PR001. The start of the Phase 1/2 trial in nGD is anticipated to be delayed approximately one quarter and to begin enrollment in the first half of 2020. The modification to the nGD Phase 1/2 trial design is not anticipated to delay the overall timeline to trial completion.

"Prevail is developing PR001 for two indications, both with urgent unmet needs," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "We are excited to begin dosing patients in our Phase 1/2 clinical trial for PD-GBA this year and are dedicated to developing PR001 for pediatric nGD, the most progressive form of Gaucher disease, which involves neurological manifestations that cause severe morbidity and mortality. We believe PR001 has tremendous potential to slow or stop disease progression in patients with PD-GBA or nGD, who currently have no disease-modifying therapeutic options."

About Prevail Therapeutics
Prevail Therapeutics is a gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases. Prevail was founded by Dr. Abeliovich in 2017, through a collaborative effort with The Silverstein Foundation for Parkinson's with GBA and OrbiMed, and is headquartered in New York, NY.

Forward-Looking Statements
Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Examples of these forward-looking statements include statements concerning: the likelihood of its interactions with the FDA to support Prevail's clinical development plans; the anticipated timing of Prevail's Phase 1/2 clinical trials of PR001 in each of its two indications; and the ability of PR001 to slow or stop disease progression in patients with PD-GBA or neuronopathic Gaucher disease. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties include, among others: Prevail's novel approach to gene therapy makes it difficult to predict the time, cost and potential success of product candidate development or regulatory approval; PR001 or Prevail's other gene therapy programs may not meet safety and efficacy levels needed to support ongoing clinical development or regulatory approval; and the regulatory landscape for gene therapy is rigorous, complex, uncertain and subject to change. These and other risks are described more fully in Prevail's filings with the Securities and Exchange Commission (SEC), including the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the period ended June 30, 2019, filed with the SEC on August 14, 2019, and its other documents subsequently filed with or furnished to the SEC. All forward-looking statements contained in this press release speak only as of the date on which they were made. Except to the extent required by law, Prevail undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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